

STATEMENT OF SUSAN L. WEINER
NORTH AMERICAN BRAIN TUMOR COALITION
BEFORE THE HEALTH AND ENVIRONMENT SUBCOMMITTEE
OF THE HOUSE COMMERCE COMMITTEE
APRIL 23, 1997
ON THE REAUTHORIZATION OF THE PRESCRIPTION DRUG
USER FEE ACT AND
REFORM OF THE FOOD AND DRUG ADMINISTRATION

Mr. Chairman and Members of the Committee, thank you for the opportunity to testify about the reform of the Food and Drug Administration (FDA). I am the Vice Chair of the North American Brain Tumor Coalition, a network of charitable organizations which represents the interests of brain tumor patients and their families.

North American Brain Tumor Coalition

The North American Brain Tumor Coalition's nine member organizations -- seven in the United States and two in Canada -- focus primarily on raising funds from private sources for the support of research related to brain tumors. Members of the Coalition have raised and awarded over \$12 million for research, and our fundraising efforts are ongoing. Some member organizations also sponsor conferences for patients and health care professionals, support graduate medical fellowships, and provide educational materials and support services to patients and families. As a Coalition, we seek to raise public awareness of the

problem of brain tumors, advocate for increased research funding and ready access to quality health care, and serve as the voice of brain tumor patients on other important issues.

I am also the mother of Adam Weiner, who died at age 13 after living his entire life with a brain tumor. I am speaking today on behalf of Adam and other brain tumor patients and their families who have been confronted with difficult -- if not impossible -- decisions about the best treatment for their illnesses. When patients receive a diagnosis of a brain tumor, the news is often devastating for them and their families. If the brain tumor is malignant, there is only a 27 percent chance of surviving five years. Furthermore, because the brain is the control center for thought, emotion, and movement, brain tumors can have a staggering impact on quality of life.

If brain tumor patients are to have a fighting chance against their disease, they and their families must have full information about the range of treatment options and must be allowed to make independent decisions about the risks they face in treatment. The same issues arise for other patients with serious and life-threatening diseases.

Cancer Leadership Council

Several of the positions I am advocating today have previously been endorsed by the Cancer Leadership Council (CLC), a group of eight patient advocacy organizations in which the North American Brain Tumor Coalition

participates.¹ It is the concerns of patients with cancer and other serious and life-threatening illnesses that guide the principles of FDA reform that I will outline.

During the last Congress, the CLC supported several legislative reforms of the FDA: a liberalization of FDA restrictions on dissemination of information on off-label drug use, similar to language in the Mack-Frist bill; literature-based approvals of supplemental new drug applications (SNDAs); and standards to provide accountability and predictability in the drug approval process.² The cancer patient community acknowledges the critical role of the FDA in ensuring the safety and effectiveness of the drugs, biologicals, and devices that we use. We have not recommended that the FDA be privatized or that it be stripped of its regulatory powers, and we never will.

Some patient advocates -- including representatives of the Patients' Coalition -- have suggested that patients speak with a single united voice in opposition to reform of FDA. Cancer patients stand apart from these patients because we do support reform of certain FDA policies. The reforms we advocate would remove limits and reduce delays in the patients' access to the best treatment

¹ The CLC includes Cancer Care, Inc.; Candlelighters Childhood Cancer Foundation; Susan G. Komen Breast Cancer Foundation; National Alliance of Breast Cancer Organizations; National Coalition for Cancer Survivorship; North American Brain Tumor Coalition; US TOO International; and Y-ME National Breast Cancer Organization.

² The CLC has not taken a position on the issue of stem cell transplants, which has arisen only recently, or incentives for pediatric research.

options. In addition, we believe that a more productive and cooperative relationship between industry and the agency is necessary if science is to realize its fullest potential in diagnosing, preventing, and curing human disease.

The debate about FDA reform is essentially about the appropriate balance between the power of FDA to regulate drugs, devices, and biological products and the desire of patients, physicians, and industry for speedier product approvals and fewer restrictions on information dissemination.

Rapid Approval of New Therapies

Some brain tumor patients measure their survival by days and weeks, so the speed with which new therapies are made available to them is a life and death matter. This is also true for many other cancer patients. We applaud the progress of the FDA, with new resources available through the Prescription Drug User Fee Act (PDUFA), in reducing the drug approval time substantially. We support the reauthorization of the User Fee Act and the goal of trimming approval times to 10 months over a five-year period. We are also pleased that FDA and industry are discussing provisions of the User Fee Act reauthorization which would reduce the clinical development phase of the drug development process.

There is a great deal of discussion about whether the reauthorization of the User Fee Act should be tied to other reform of FDA. We believe the Prescription Drug User Fee Act was a major reform of the FDA because it required the agency to meet defined timelines for drug approval and compelled the industry

to help cover the costs of FDA's valuable contribution to their products. We believe it is logical, appropriate, and efficient to link the user fee reauthorization to FDA reform.

We also support the President's Anticancer Initiative, announced and implemented in the spring of 1996, which provides for the accelerated approval of cancer drugs using surrogate endpoints, such as measurable tumor shrinkage in place of improved survival or complete tumor shrinkage. This initiative, combined with the priority review of some cancer drugs, has resulted in the recent approval of several important cancer agents, including one for use in the treatment of brain tumors, and essentially eliminated the backlog of cancer drug applications at the agency.

We believe the anticancer initiative was the result, at least in part, of Congressional pressure for reform. We are focused now on how to guarantee that the FDA makes further strides toward reducing the approval time for all cancer therapies, broadly defined to include diagnostics, devices, and palliative treatments.

Supplemental New Drug Applications

We agree with those who want to bring product labels up-to-date by adding off-label uses to the approved labeling. This can be accomplished by streamlining the process for reviewing supplemental new drug applications (SNDAs). The CLC has urged that FDA be required to accept peer-reviewed

literature reports of clinical trials to support approval of secondary uses of

approved drugs, biologics, or devices and has criticized FDA for its failure to act on this issue.

FDA has recently addressed the approval of SNDAs in a Guidance for Industry, "FDA Approval of New Cancer Treatment Uses for Marketed Drug and Biological Product." While we are pleased that FDA has finally issued a draft policy on SNDAs, the document does not modify FDA policy substantially and does not appear to simplify or speed up the process for approval of SNDAs. Our goal is simply to expedite the approval of SNDAs by allowing the agency to approve SNDAs on the basis of peer-reviewed literature.

At a Congressional hearing in the fall of 1996, Acting Commissioner Friedman, who was then a Deputy Commissioner, stated, "[F]or products that lack marketing exclusivity and for supplemental indications that benefit only small populations, no combination of strategies reliably will induce commercial sponsors to pursue supplemental applications. To get these uses into labeling likely will require allocation of public funds, particularly where additional data would have to be developed to demonstrate that a use is safe and effective." Commissioner Friedman has identified a serious hurdle to keeping labels updated. We urge FDA to consider seriously Commissioner Friedman's proposal and also to reevaluate its position on literature-based reviews. Legislation may be necessary to establish the standards for approval of SNDAs and to authorize incentives for filing certain SNDAs.

Dissemination of Information on Off-Label Use

Even if improvements are made in the approval of SNDAs, however, cancer treatment changes so rapidly that labels will never be completely up-to-date. More than half of all cancer chemotherapy consists of off-label use, and streamlining the SNDA process will not eliminate the use of cancer drugs off-label. Patients and physicians have a right to know the very latest information about cancer chemotherapy regimens, and the FDA policy restricting dissemination of this information does not always serve the best interests of patients. Therefore, the cancer patient community continues to oppose current FDA policy restricting information dissemination.

In January of 1996, 80 national cancer organizations, including the North American Brain Tumor Coalition, wrote to FDA asking the agency to change its position to allow pharmaceutical sponsors to disseminate to physicians peer-reviewed articles and textbooks discussing off-label use. FDA has not modified its position on this issue, and we still support legislative reform of FDA policy in this area. We urge the Committee to include in its bill provisions on information dissemination similar to those in the Mack-Frist legislation of the 104th Congress.

Incentives for Research on Pediatric Uses of Drugs

We recognize that the FDA cannot solve all the problems facing patients with serious and life-threatening illnesses. In the case of brain tumors, the slow and incremental progress toward improved therapies is limited primarily by the

state of the science on brain tumors, and progress will be made only through cooperation among academic researchers, industry, and public sector regulators and researchers.

As Executive Director of the Children's Brain Tumor Foundation, I am particularly concerned about the issue of how best to treat children with serious illnesses. These children present great challenges to their physicians and to researchers, and special efforts may be necessary to meet their needs. For example, children with brain tumors may metabolize drugs differently than adults, and therapies may affect brain and other organ development and therefore have a profound influence on a child's cognitive and emotional development. The pediatric use of drugs or other aggressive therapies tested only in adults also raises serious ethical issues. However, these drugs may represent the only treatment option for seriously ill children, and their use may be absolutely necessary.

Some have suggested that companies be mandated to conduct pediatric trials at the same time they are conducting trials of drugs in adults. We believe this requirement could have the effect of impeding research on therapies for both adults and children, and we recommend instead that incentives be provided to pharmaceutical companies to conduct pediatric drug trials. The North American Brain Tumor Coalition supported the pediatric research bill introduced by Senator Nancy Kassebaum in the last Congress. This legislation would have provided a period of six months of additional exclusivity to a company if it conducted pediatric

studies of a drug either prior to approval or postmarketing. We believe the approach of the Kassebaum bill is consistent with the policy, outlined by Commissioner Friedman and described above, of providing incentives where the supplemental indication benefits only small populations or where there is no market exclusivity.

Some have suggested that the six-month period of exclusivity is an inadequate incentive to companies to conduct pediatric clinical trials. Although we do not feel confident in making a judgment regarding the precise term of additional exclusivity that should be granted, we believe legislation similar to the Kassebaum bill should be included in the user fee/FDA reform bill. In addition, we support federal financial support, through National Institutes of Health (NIH) grants, for pediatric research. The combination of incentives to the private sector and federally-sponsored research would markedly improve the state of knowledge about treatment options for pediatric patients.

Stem Cell Regulation

The cancer community is perplexed and very concerned about the FDA plan to regulate stem cell transplants. Based on the experience of brain tumor patients and reports from the transplant physicians with whom we work, stem cell transplants are safe and effective and there is no apparent problem that needs to be addressed by FDA regulation. FDA has nevertheless announced plans to create a new regulatory system for tissue culture, including stem cells used in a variety of

procedures known as stem cell transplantation. Over the course of the

past decade, great progress has been made in the use of these medical procedures to combat a variety of cancers, including brain tumors.

The FDA has stated its intention to regulate these procedures -- performed by individual physicians on their own patients -- as though they were investigational biological products. We understand that a new industry that is engaged in the collection, manipulation, and storage of stem cells has developed to facilitate transplantation procedures. We support the regulation of these entities which are involved in the preparation of stem cells on a commercial scale for many different patients. However, there is reason to question the extension of that regulation to the individual physician's practice of medicine.

Cancer patients are concerned that the proposed regulations, by requiring INDs for each new procedure and eventually a biologic license application and approval, would seriously inhibit the development of this important technology. Specifically, the regulations could: 1) restrict our access to stem cell transplants because some institutions will not continue to perform them; 2) result in life-threatening delays while physicians receive approval to modify their licensed stem cell procedure in order to meet a particular patient's needs; 3) create reimbursement difficulties if stem cell transplants are considered experimental because they are subject to an IND; and 4) slow the progress of research because transplanter will have to receive FDA approval for new, revised, or improved procedures.

In our view, stem cell transplants are a research “success story.”

These transplants are being conducted through clinical trials until their safety and effectiveness are established, and then they are becoming part of the care offered by practicing oncologists. The FDA regulatory scheme threatens that successful system. We believe this is an area where Congressional intervention may be necessary if FDA continues to pursue this proposed regulatory scheme.

Responsiveness to the Needs of Patients

The efforts of the FDA to improve its communications with the cancer patient community are commendable. Many in the cancer patient community have been involved in the FDA Cancer Liaison Program’s activities, including recruitment of representatives to serve on FDA advisory committees, the effort to list clinical trials on the Physician Data Query Database, and educational programs for survivors and other advocates. We intend to continue our relationship with the FDA and this office.

We look forward to continued improvements, through regulatory and legislative efforts, in the approval of cancer drugs and the availability of information about all treatments.

Thank you for inviting me to appear before the Committee and for your attention to my testimony.

